

National Organization for Rare Disorders, Inc.®

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... out of the darkness,
into the light ...

MEMBER ORGANIZATIONS

Alpha One Antitrypsin Deficiency
National Association
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AHO Association
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Dan Laryngeal Papilloma Foundation
American Porphyrria Foundation
American Syringomyelia Alliance Project
Aplastic Anemia Foundation of America
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United Leukodystrophy Foundation, Inc.
United Mitochondrial Disease Foundation
VHL Family Alliance
Wegener's Granulomatosis Support
Group, Inc.
Williams Syndrome Association
Wilson's Disease Association

May 16, 2001

Sandra Titus
Food and Drug Administration
Center for Drug Evaluation and Research (HFD-21)
5600 Fishers Lane
Rockville, MD 20857

Dear Ms. Titus:

I will be appearing at the June 6, 2001 FDA Advisory Committee meeting for sodium oxybate, a treatment for narcolepsy and cataplexy. Attached is a written history of the drug for use by committee members. I will confine my oral comments to the lessons we have learned from restricted distribution systems for Clozaril and Thalidomide.

I look forward to seeing you on June 6.

Very truly yours,

Abbey S. Meyers
President

ASM:aa

Acid Maltase Deficiency Association
ALS Association/Greater Philadelphia
Chapter
American Autoimmune Related Diseases
Association
American Behcet's Disease Association,
Inc.
American Self-Help Clearinghouse
Angel view Crippled Children's
Foundation
Ataxia Telangiectasia Children's Project
CDGS Family Network
Canadian Organization for Rare Disorders

Children's Living with Inherited Metabolic
Diseases
Children's Medical Library
Children's PKU Network
Chromosome Deletion Outreach, Inc.
Chronic Granulomatous Disease
Association, Inc.
Consortium of Multiple Sclerosis Centers
Contact A Family
Cooley's Anemia Foundation
Cushing Support & Research Foundation
Family Caregiver Alliance
Family Support System for North Carolina

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Les Turner ALS Foundation, Inc.
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Elasticum
National Gaucher Foundation
National Lymphedema Network
National Niemann-Pick Disease
Foundation

National Patient Air Transport Helpline
National Spasmodic Dysphonia Association
Organic Acidemia Association
Osteoporosis and Related Bone Diseases
National Resource Center
Parents Available to Help (PATH)
Parent to Parent of New Zealand
Rare and Expensive Disease Management
Program
Recurrent Respiratory Papillomatosis
Foundation
Restless Legs Syndrome Foundation
Sarcoid Networking Association

Shwachman Syndrome Support Group
Sickle Cell Disease Association of Texas
Gulf Coast
Society For Progressive Supranuclear
Palsy, Inc.
Sotos Syndrome Support Association
Takayasu's Arteritis Association
Taiwan Foundation for Rare Disorders
Treacher Collins Foundation

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Williams Syndrome Association
Wilson's Disease Association

Testimony by
Abbey S. Meyers, President
National Organization for Rare Disorders (NORD)

Regarding
Xyrem (sodium oxybate), Orphan Medical Inc.

Before the
FDA Peripheral & Central Nervous
System Drugs Advisory Committee

June 6, 2001

A TWENTY YEAR SAGA

Sodium Oxybate was one of the original therapeutic compounds that led to enactment of the *Orphan Drug Act of 1983*. Its value in treating the most devastating symptoms of Narcolepsy, known as Cataplexy, has been known since the late 1970s. Even after the law was enacted, no company was willing to develop the drug for the commercial market because they believed it would not be profitable enough.

During the 1980s, the FDA's Office for Orphan Products Development funded a research grant to an academic scientist for a small clinical trial of sodium oxybate. After several years, he published the study, which raised the expectations of the narcolepsy community. Still no company was interested. We turned to the generic drug industry, and a generic manufacturer agreed to adopt the drug. He spent about five years stabilizing the compound but did not launch a new clinical trial. Finally that company was merged with another, so FDA again sought a new sponsor. Orphan Medical stepped in where no other company was willing to tread.

About that time, the drug began to appear in health food stores with bogus muscle building claims. But the one thing sodium oxybate does very well is put people to sleep. When young people started arriving at emergency rooms, doctors realized they were in a deep sleep, and they started raising warnings. FDA eventually ordered the supplement off the market when it became associated with the "date rape" drugs. DEA wanted to make it illegal for all uses, without regard to its valid medical use for narcolepsy. We pointed out that none of the illegal use of the drug was associated with the pharmaceutical formulation, and instructions for making sodium oxybate are on the Internet. Therefore, the FDA and DEA cannot stop use of the compound unless they take the instructions off the Internet.

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Dedicated to Helping People with Orphan Diseases

Now after more than 20 years, the studies are done and Orphan Medical has submitted an NDA for approval of sodium oxybate for narcolepsy and cataplexy. People with the most severe form of narcolepsy need this orphan drug desperately. The question is safety of a distribution process that will assure it gets into the hands of patients who need it, and not to the young people who will use it for the wrong purpose.

Keep in mind that people with narcolepsy currently struggle with an inequitable distribution system for amphetamines. If they need Ritalin or Dexedrine, they usually have to tell their pharmacist days in advance because most pharmacies do not want to stock those drugs. They cannot order amphetamines through the mail, and in many states they are forced to see their doctor more than medically necessary in order to get new prescriptions. It is not easy to have a disease that is treated with medicines that have potential for abuse.

I submit that safe distribution systems can be implemented, notwithstanding the Internet. Unless law enforcement and Congress are willing to take the information off the World Wide Web, those who misuse sodium oxybate will be able to continue manufacturing it in their kitchen sink. We already have good models for controlled distribution of prescription drugs, and these are the models that this committee should consider.

The best model is probably thalidomide, a drug that matches no other in the history of medicine in terms of horror, but is nevertheless an approved orphan drug on the American market today. Doctors who prescribe it, and pharmacies that dispense it, register with the manufacturer so that every pill can be monitored and traced in the distribution system. Another important drug is Clozaril for schizophrenia. That drug also is carefully distributed through registered pharmacies, and patients have to prove that they received a satisfactory blood test before their next weekly prescription is dispensed. Our primary concern about these systems is that manufacturers should not be privy to patients' names and addresses. An independent party should guard personally identifiable information.

Both Thalidomide and Clozaril have been approved by the FDA and successfully marketed in the United States even though their distribution is tightly controlled. For serious diseases that do not respond to other therapies, it is incumbent on FDA to find safe ways to get the treatments to the patients who need them. Narcolepsy with cataplexy is a very serious disease, as dangerous as epilepsy because patients lose consciousness suddenly and uncontrollably. We know the most important rule of medicine is, "First, do no harm". To deny this drug to people with cataplexy will do harm to them. We ask you to allow this drug to get to market with a carefully controlled distribution system so we can put this nightmarish saga of sodium oxybate behind us and let these patients get back to living productive lives.

Thank you.